Taking gene therapy from laboratory to the clinic, beyond a gene therapy product

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Introduction

Taking gene therapy from the laboratory to the clinic involves many factors that we usually neglect; in this review I will try to explain it using an example, the gene therapy for Wiskott-Aldrich syndrome. With more communication and information about gene therapy it would be easier for society to accept that gene therapy is not something to fear, so I created a website with educational resources and a forum in order to make people understand what gene therapy really is.

Objectives

- To study in which moment are gene therapies large scale production for blood disease with the example of the Wiskott-Aldrich syndrome (WAS).
- To create a website for patients affected by rare diseases that could be susceptible of receiving gene therapy.
- To create a forum in order to promote opinion exchange between website users. No registration is needed.

Website

http://xavierrivera9.wix.com/gene-therapy1

- Videos to understand what gene therapy is.
- Related experiences

- Information about basic concepts of gene therapy and clinical trials

- Link to the forum, a space to exchange opinions and points of view

A real life example, gene therapy for Wiskott-Aldrich Syndrome

Learn about the disease
Get money for the project
Get approval for the project
Perform clinical research
Perform biological research

- How many corrected cells will I need to reach in a patient for a clinical benefit
- Limits in gene transfer methodologies
- good animal model to test both the efficacy & safety
- A good antibody to track the re-expression of the missing protein

Design a gene therapy.
Get money and approval for clinical trials

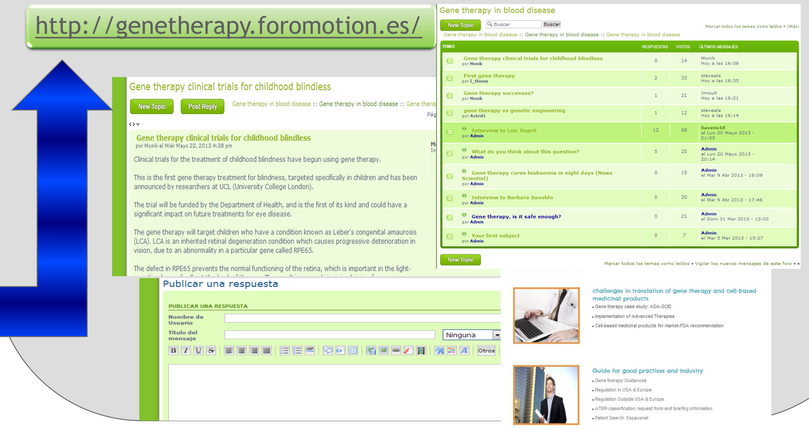
- A good correction of T lymphocyte function
- Selective advantage of T modified lypmhocites.

Perform clinical trial
Get approval for general clinical use

- An international phase I/II clinical trial for Wiskott-Aldrich Syndrome (WAS) is performed in Genethon (France)

FORUM

http://genetherapy.foromotion.es/



Interviews

Barbara Savoldo, a doctorate who works in the Texas Children Cancer Center

Dr Francesc Solé, responsible of the research group in SMD of the Josep Carreras Research Institute against leukaemia

Loïc Dupré, researcher in INSERM Toulouse who worked in Telethon Institute for Gene therapy in Milan.

To work in hematologic research you need a lot of enthusiasm, optimism, patience and to get down to work. But at the end of the hard work you will get your compensation in the form of results. (Dr Solé)

We initially obtained a very good correction of T lymphocyte function by means of WAS gene transfer with retroviral vectors. (Dr Dupré)

I believe the secret to translate the lab discoveries into clinical trials is to have a great team of specialists (MDs, scientists, regulatory affairs support, GMP trained support, research coordinators, nurses) and someone with a vision to coordinate all of them. (Dr Savoldo)

Conclusion

A dialog between all parts involved in gene therapy is needed, with more information gene therapies would be more respected. That would increase investments and finally gene therapy would become an habitual clinical practise.

I encourage everybody interested in gene therapies to visit the website (http://xavierrivera9.wix.com/gene-therapy1) and add comments to the forum.

Nowadays it is indispensable to scale up the production of gene therapies because the diseases that we would be able to cure using a gene therapy would increase exponentially with new approaches.
All the information of the steps involved in the application of a gene therapy to the Wiskott-Aldrich syndrome is more specifically detailed in the website.

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